3Q21 Financial Results Conference Call & Webcast

November 9, 2021

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product or to treatment sites. In addition to the impact of the COVID-19 pandemic, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, UK, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies, commercialization and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding corporate financial guidance and financial goals and the attainment of such goals and statements regarding projections of the Company's revenue and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2020, and on Form 10-Q for the quarter ended September 30, 2021, to be filed today. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

<u>Non-GAAP Financial Measures</u>

In addition to financial information prepared in accordance with U.S. GAAP, this presentation also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

A RARE COMPANY

Amicus has built a leading, fully integrated, global rare disease biotechnology company

Galafold® (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

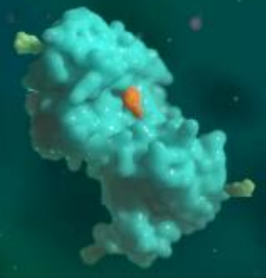
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Registration in Pompe Disease



GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

50+ Lysosomal Disorders and More Prevalent Rare Diseases

$557M Cash

as of 9/30/21

Two Clinical-Stage Gene Therapies

Amicus Therapeutics

2021 Key Strategic Priorities

1. **Achieve double-digit Galafold growth and revenue of $300M to $315M**
2. **Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals**
3. **Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline**
4. **Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs**
5. **Maintain strong financial position**

Caritas Strategic Rationale

On-Track to Complete Planned Business Combination of Amicus Gene Therapy Business with ARYA IV Resulting in the Launch of Caritas Therapeutics in Late 2021/Early 2022

- **Enhancing the ability of both Amicus and Caritas to meet the unmet needs of patients living with rare diseases**
- **Accelerating Amicus' path to profitability, expected in 2023**
- **Significantly strengthening the financial profile of each company**
- **Accelerating the development and broadening the scope of our gene therapy portfolio**
- **Reinforcing management focus on key strategic and financial goals**
- **Unlocking value while creating a more targeted investment thesis for shareholders**

Galafold® (migalastat) Global Launch…

… taking a leadership role in the treatment of Fabry disease

“We push ideas as far and as fast as possible”
- Amicus Belief Statement

Galafold Snapshot (as of September 30, 2021)

Galafold is an orally delivered, small molecule precision medicine with a unique mechanism of action for Fabry patients with amenable variants that replaces the need for intravenously delivered ERT

One of the Most Successful Rare Disease Launches

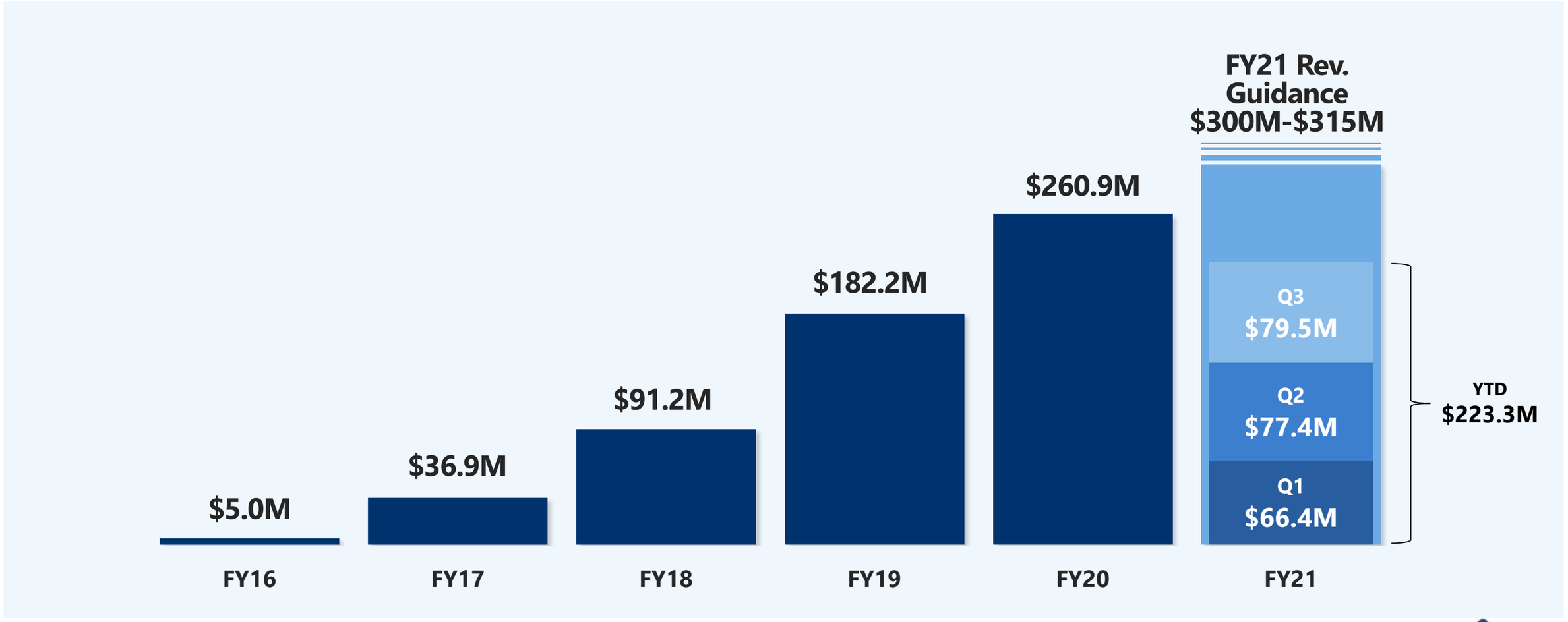
- **$79.5M +18% YoY** — 3Q21 Galafold Revenue
- **$300M-$315M** — FY21 Global Galafold Revenue
- **1,384** — Amenable Mutations Included in the EU Label
- **348** — Amenable Variants in U.S. Label
- **Continued Expansion in 2021**
- **40+** — Countries with Regulatory Approvals: Including U.S., EU,U.K., Japan and Other Countries

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/Galafold.pdf. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

Amicus Therapeutics

Galafold Success and FY21 Revenue Guidance

Galafold momentum remains on track to deliver within full year 2021 revenue guidance

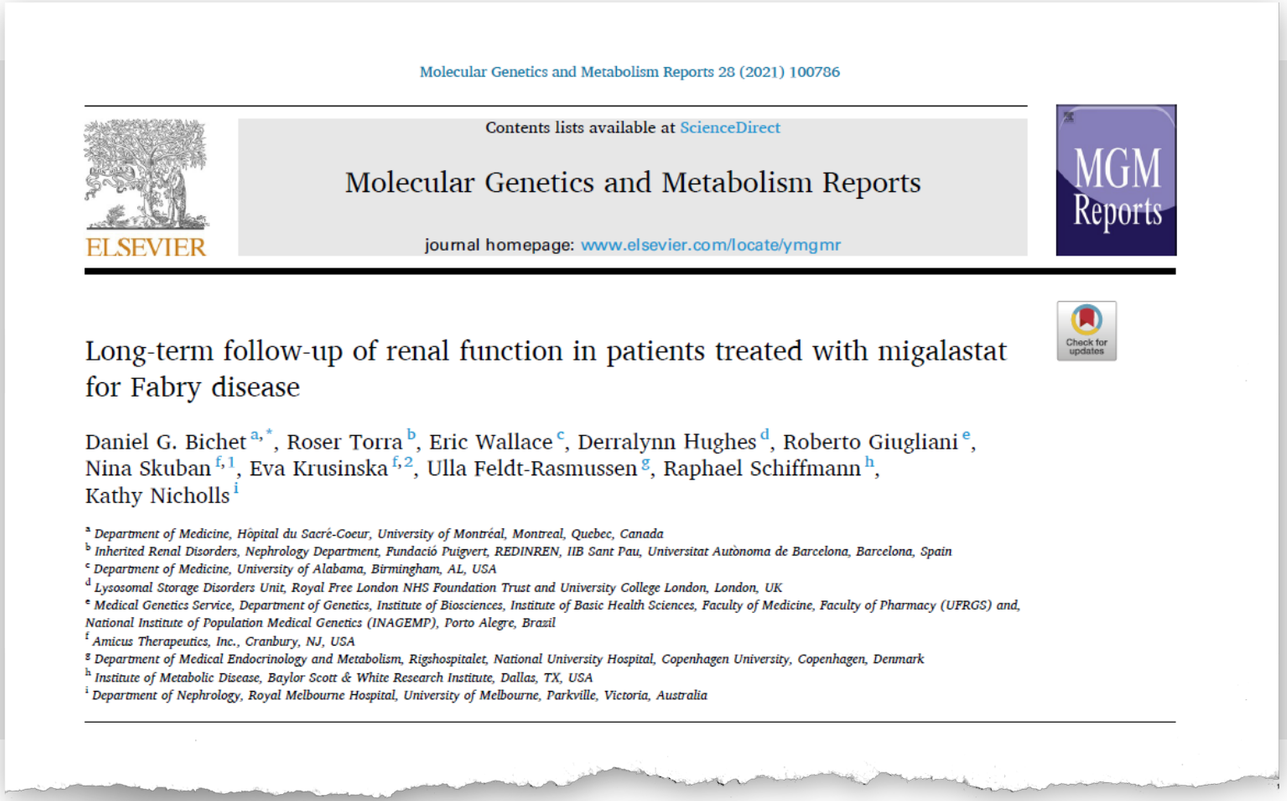


Galafold Long-Term Treatment Publication

Published in the September 2021 Issue of *Molecular Genetics and Metabolism Reports* showing generally stable renal function during long-term treatment

Key Findings[1,2]

- Migalastat-treated patients had generally stable renal function for up to 8.6 years
- Migalastat stabilized eGFR in ERT-naive and ERT-experienced males and females compared to historical untreated controls
- Migalastat generally stabilized eGFR in ERT-naïve male patients with the classic phenotype



Molecular Genetics and Metabolism Reports 28 (2021) 100786

Contents lists available at ScienceDirect

Molecular Genetics and Metabolism Reports

journal homepage: www.elsevier.com/locate/ymgmr

Long-term follow-up of renal function in patients treated with migalastat for Fabry disease

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1. Study limitations: Post hoc design, small sample sizes in some subgroups, lack of statistical comparisons with untreated or ERT-treated historical cohorts, assessment of renal function using eGFR, and the heterogeneity of statistical methods used to estimate eGFR slopes in the literature limited direct comparisons.
2. The study includes data that are not in the FDA-approved Prescribing Information (PI) for Galafold and the clinical relevance of the changes in eGFR in the treatment of Fabry disease has not been established.

Outlook for 2021

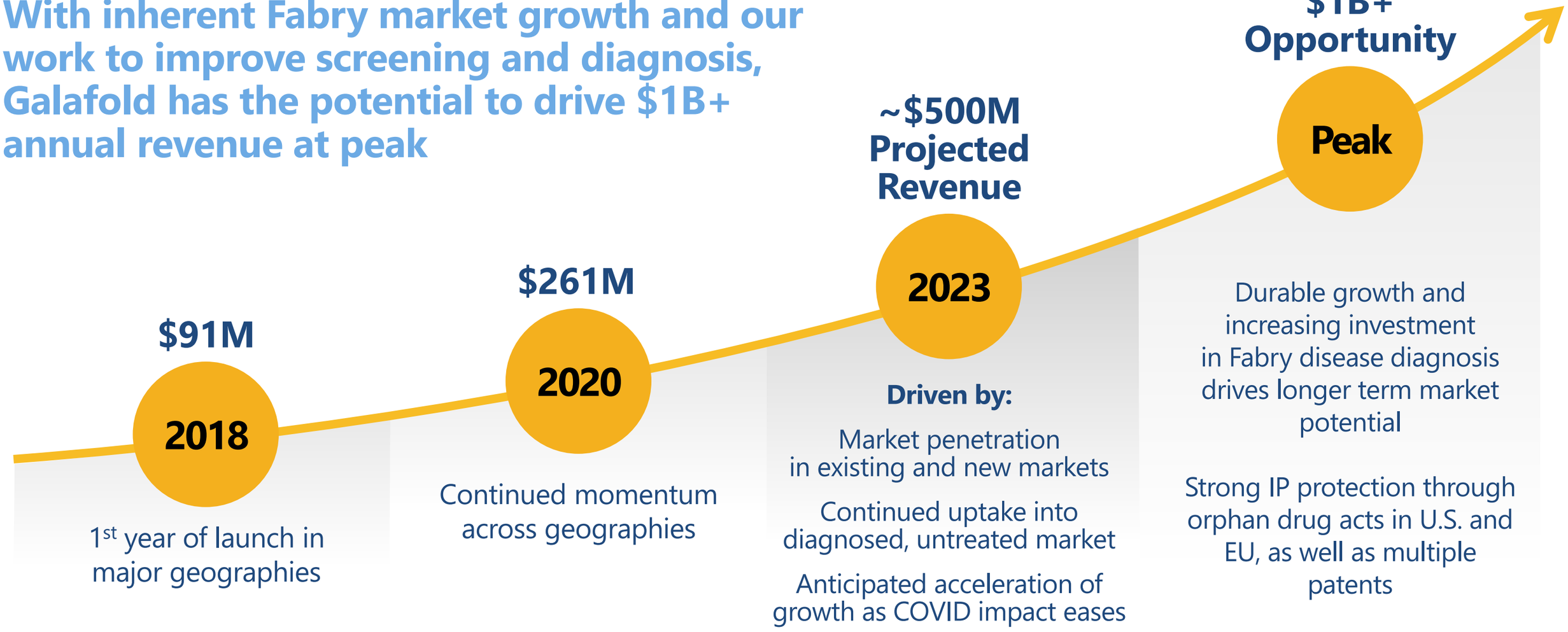
Continued double-digit Galafold revenue growth to within $300M-$315M in 2021

Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success

- Global demand remains strong with 3Q21 revenue growth rate of 18% and continued growth anticipated in 2021 and beyond
- In 2021, project double-digit revenue growth with net new patient starts expected to be greater than in 2020
- Expanded EU label following the European Commission approval for use in adolescents
- COVID continues to impact time between patient identification and treatment initiation
- Expect higher patient adds and revenue growth in 2H2021
- Continue to see >90% compliance and adherence rates globally

Galafold Opportunity

With inherent Fabry market growth and our work to improve screening and diagnosis, Galafold has the potential to drive $1B+ annual revenue at peak

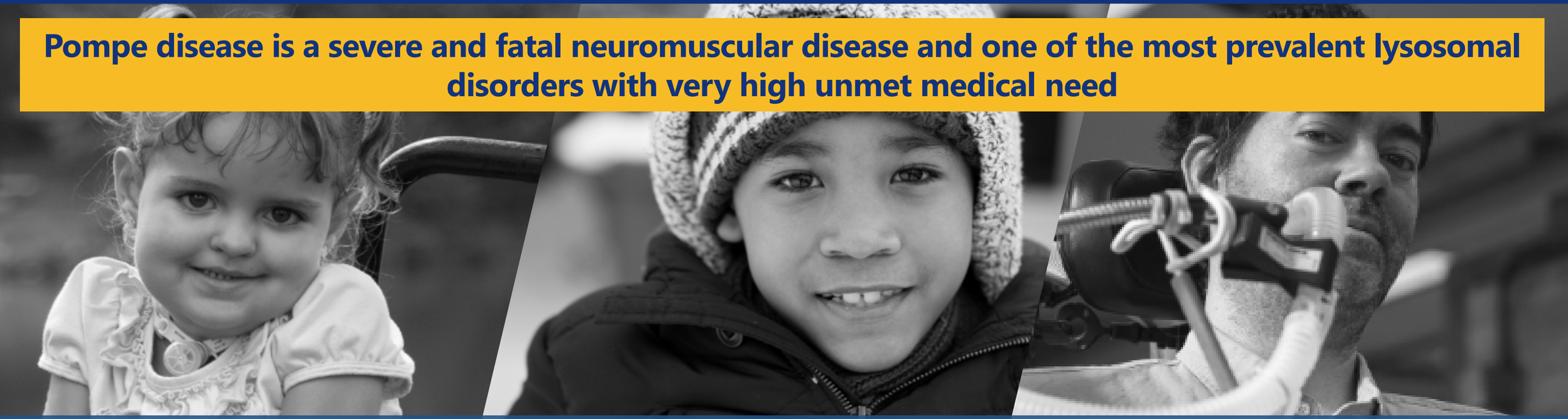


AT-GAA: Next Potential Standard of Care for Pompe Disease

"We encourage and embrace constant innovation"
- Amicus Belief Statement

Pompe Disease Overview

Pompe disease is a severe and fatal neuromuscular disease and one of the most prevalent lysosomal disorders with very high unmet medical need



5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis

Age of onset ranges from infancy to adulthood

Patients on current standard of care decline after ~2 years

Respiratory and cardiac failure are leading causes of morbidity and mortality

Deficiency of GAA leading to glycogen accumulation and cellular dysfunction

Symptoms include muscle weakness, respiratory failure and cardiomyopathy

~$1.1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on year ending June 30, 2021. Source: Sanofi Press Releases

Phase 3 PROPEL Study

Primary, Key Secondary and Biomarker Endpoint Heat Map

Endpoints across motor function, pulmonary function, muscle strength, PROs and biomarkers favored AT-GAA over alglucosidase alfa

		Overall population				ERT-experienced			
	Endpoints	Cipaglucosidase alfa/miglustat n=85		Alglucosidase alfa/placebo n=37		Cipaglucosidase alfa/miglustat n=65		Alglucosidase alfa/placebo n=30	
		Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)	Baseline, mean	CFBL at week 52, mean (SE)
Motor function	6MWD, m	357.9	20.8 (4.6)	351.0	7.2 (6.6)	346.9	16.9 (5.0)	334.6	0.0 (7.2)
	GSGC total score	14.5	-0.5 (0.3)	14.5	0.8 (0.3)	15.6	-0.5 (0.3)	15.5	0.6 (0.4)
	10-meter walk, s	9.7	-0.5 (0.6)	9.6	1.9 (1.0)	10.4	-0.6 (0.9)	10.2	2.5 (1.2)
	4-stair climb, s	14.1	-8.5 (7.9)	8.2	0.3 (1.0)	17.3	-11.1 (10.5)	9.3	0.6 (1.2)
	Gower's maneuver, s	10.8	-0.3 (0.7)	19.8	-2.2 (1.4)	11.5	-0.4 (0.8)	23.9	-2.6 (1.9)
	Rising from chair, s	13.6	-10.2 (9.7)	4.5	-0.5 (0.7)	17.6	-13.7 (13.0)	5.2	-0.4 (0.9)
Pulmonary function	FVC, % predicted	70.7	-0.9 (0.7)	69.7	-4.0 (0.8)	67.9	0.1 (0.7)	67.5	-4.0 (0.9)
	MIP, % predicted	61.8	2.1 (2.1)	59.9	-2.7 (2.8)	61.3	1.0 (2.5)	55.0	-1.7 (1.5)
	MEP, % predicted	70.7	0.6 (2.4)	65.1	-1.6 (2.1)	70.7	-2.7 (2.7)	62.2	-3.9 (1.8)
Muscle strength	Lower MMT score	28.0	1.6 (0.4)	27.7	0.9 (0.4)	26.4	1.6 (0.5)	26.1	0.9 (0.5)
	Upper MMT score	34.3	1.5 (0.4)	34.7	0.7 (0.6)	33.7	1.8 (0.4)	34.2	0.4 (0.7)
	Total MMT score	62.3	3.1 (0.7)	62.4	1.4 (0.8)	60.1	3.4 (0.9)	60.3	1.1 (0.9)
PROs	PROMIS®-Physical Function	66.9	1.9 (0.8)	68.0	0.2 (1.8)	64.4	1.8 (0.9)	66.9	-1.0 (2.0)
	PROMIS®-Fatigue	22.3	-2.0 (0.6)	21.1	-1.7 (1.1)	22.0	-1.9 (0.7)	20.4	-0.3 (1.0)
Biomarkers	Urine Hex4, mmol/mol	4.6	-1.9 (0.3)	6.9	1.2 (0.7)	4.6	-1.7 (0.3)	7.2	1.9 (0.8)
	Serum CK, U/L	447.0	-130.5 (25.1)	527.8	60.2 (26.2)	441.8	-118.0 (28.4)	492.3	79.6 (26.9)

Based on LOCF means

Treatment group favored

Nominal statistical significance ($P<0.05$)

Note: * Nominal P-value <0.05; based on LOCF means

Source: Presented at the *16th International Congress on Neuromuscular Diseases (ICNMD)* May 2021.

Amicus Therapeutics

AT-GAA: Highlights

AT-GAA for Pompe Advances Toward Approval

- U.S. FDA accepted for review the BLA and NDA for AT-GAA
- MAA for AT-GAA submitted with the EMA
- Granted positive scientific opinion through the Early Access to Medicines Scheme (EAMS) by the United Kingdom's MHRA
- 150+ patients worldwide now being treated with AT-GAA, including adults, adolescents and infants
- Pediatric study for adolescents up to 17 years with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile-onset disease expected to begin this year
- Expanded access program for infantile- and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered.

Next-Generation Gene Therapy Platform

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

Harnessing the Power of Genetic Medicine

The mission of Caritas is to transform the lives of children and adults living with rare genetic diseases through advanced protein engineering and innovative vector technologies

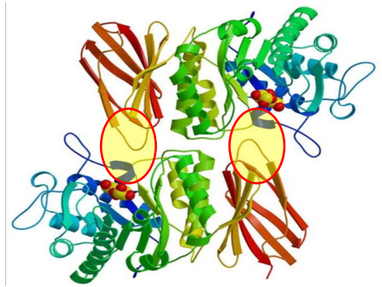
CARITAS THERAPEUTICS

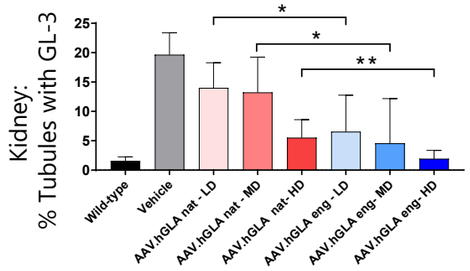
1. **Diversified portfolio of clinical and preclinical rare disease development programs with pivotal study start in CLN3, three INDs, and two IND candidates targeted through end of 2023**
2. **Proprietary platform technologies and protein engineering capabilities enabling innovative, clinically differentiated gene therapies**
3. **Exclusive relationship with Penn to develop gene therapies for rare genetic disorders**
4. **Fully designed, ready-to-build, state-of-the-art clinical manufacturing facility with commercial expansion capabilities**
5. **Attractive risk and cost-sharing partnership with Amicus provides access to an established orphan disease commercial platform**
6. **Experienced public company leadership coupled with fully built out gene therapy discovery, research and development team**

Protein Engineering Expertise & Technologies for Gene Therapy

Differentiated gene therapy approach for greater potency and optimized cross correction through transgene engineering for stability and targeting

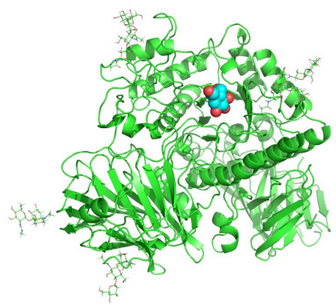
Fabry Gene Therapy

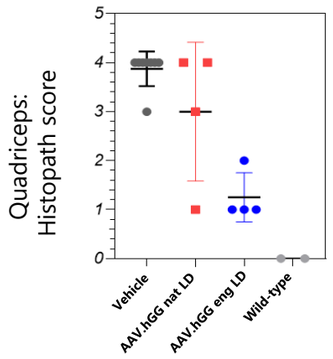




- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGLA transgene at dimer interface designed for improved stability and optimized cross correction
- Preclinical data demonstrate robust substrate reduction across all Fabry disease relevant tissues, including first evidence of dorsal root ganglia storage reduction
- IND expected in 2H2022

Pompe Gene Therapy





- Proprietary AAV capsid
- Pantropic capsid and ubiquitous promoter
- Engineered hGAA transgene with cell receptor binding motif designed for improved uptake and optimized cross correction
- Preclinical data demonstrate robust glycogen reduction in all key Pompe disease relevant tissues, including reduction in neurons of central nervous system
- IND-enabling work underway

Amicus and Caritas to co-develop the Fabry and Pompe gene therapies

Note: Data from studies in KO GLA and GAA mice

Amicus Therapeutics

Financial Summary

“We are business led and science driven”

- Amicus Belief Statement

3Q21 Select Financial Results

3Q21 revenue of $79.5M and growth rate of 18% primarily from global Galafold sales

(in thousands, *except per share data)*	**Sep. 30, 2021**	**Sep. 30, 2020**
Product Revenue	$79,545	$67,437
Cost of Goods Sold	11,696	8,399
R&D Expense	59,333	70,419
SG&A Expense	46,107	37,850
Changes in Fair Value of Contingent Consideration	3,288	1,034
Depreciation and Amortization	1,520	2,496
Loss from Operations	(42,399)	(52,761)
Income Tax Benefit (Expense)	182	(727)
Net Loss	(50,294)	(64,011)
Net Loss Per Share	(0.19)	(0.25)

Financial Outlook: Key Takeaways

- Reaffirming full-year Galafold revenue guidance of $300 million to $315 million
- Non-GAAP operating expense guidance for 2021 is expected to remain flat at $410 million to $420 million
- Balance sheet further strengthened with a ~$200 million private investment from leading biotechnology investors
- Current cash position is sufficient to achieve self-sustainability and profitability by 2023

Closing Remarks

"We believe in our future to build long-term value for our stakeholders"

- Amicus Belief Statement

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®

Appendix

Reconciliation

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Total operating expenses - as reported GAAP	**$ 110,248**	**$ 111,799**	**$ 331,033**	**$ 350,851**
Research and development:				
Share-based compensation	3,775	8,626	13,232	17,241
Selling, general and administrative:				
Share-based compensation	8,066	7,282	30,699	19,671
Changes in fair value of contingent consideration payable	3,288	1,034	4,780	2,680
Depreciation and amortization	1,520	2,496	4,691	6,299
Total operating expense adjustments to reported GAAP	16,649	19,438	53,402	45,891
Total operating expenses - as adjusted	$ 93,599	$ 92,361	$ 277,631	$ 304,960